

## Stories of Hope: Sickle Cell Disease (2014)

Español



**Adrienne Shapiro** pledged she would give her daughter Marissa the best possible life she could have—wearing herself out if necessary. Her baby girl had sickle cell disease, an inherited disorder in which the body's oxygen-carrying red blood cells become crescent shaped, sticky, rigid, and prone to clumping—blocking blood flow.

Doctors warned Adrienne that Marissa might not live to see her first birthday. When Marissa achieved that milestone, they moved the grim prognosis back a year, and then another year, and then another. Adrienne worked tirelessly to help Marissa. "I was constantly asking questions," Shapiro says. And for a long time, it worked.

However, things began to unravel for Marissa as she reached adulthood. A standard treatment for sickle cell disease—and the excruciating pain caused by blocked blood vessels—is regular blood transfusions. A transfusion floods the body with healthy, round red blood cells, lowering the proportion of the deformed, 'sickle-shaped' cells. But when she was 20, a poorly matched blood transfusion triggered a cascade of immune problems. Later, surgery to remove her gall bladder set off a string of complications and her kidneys shut down temporarily. After that, her immune system couldn't take any more. Now, at age 36, she's hypersensitive. "She can't be transfused. She can't even have tape next to her skin without her body reacting," Adrienne said.

Pain control is the newest and continuing nightmare. Adrienne tells harrowing stories of long waits in hospital emergency rooms while her daughter suffers, followed by maddening arguments with staff reluctant to provide enough drugs to control the intense pain when her daughter is finally admitted. "When she was a kid, everyone wanted to make her feel good," Adrienne says. "But when we moved from the pediatric side to the adult side, they treated her as a drug seeker and me as an enabler. It's such a slap in the face."

For Adrienne, the story is all too familiar. She is the third generation in her family with a sickle cell child. Another daughter, Casey Gibson, does not have the disease but carries the sickle cell mutation, meaning she could pass it to a child if the father also has the trait. One in 500 African Americans has sickle cell disease, as do 1 in 36,000 Hispanic people.

There is only one sure way to stop this story from repeating for generations to come, Adrienne says, and that's research. She believes stem cell science will be the answer.

"I've been waiting for this science to get to the point where it had a bona fide cure, something that worked. Now we're actually nearing clinical trials. It's so close." In fact a CIRM-funded project led by Don Kohn, MD at UCLA has been given approval by the FDA to start a clinical trial. Kohn and his team intend to remove bone marrow from the patient and fix the genetic defect in the blood-forming stem cells. Then those cells can be reintroduced into the patient to create a new, healthy blood system.

"Stem cells are our only hope," Adrienne continues, "It's my true belief that I'm going to be the last woman in my family to have a child with sickle cell disease. Marissa's going to be the last child to suffer, and Casey is going to be the last one to fear. Stem cells are going

to fix this for us and many other families."

For more information about CIRM-funded sickle cell disease research, visit our fact sheet.

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**Source URL:** <http://www.cirm.ca.gov/our-progress/stories-hope-sickle-cell-disease-2014>